

Agenda for the Public Workshop on Cell and Gene Therapy Clinical Trials in Pediatric Populations

November 2, 2010 - 8:00 AM-5:30 PM
Bethesda North Marriott Hotel and Conference Center
5701 Marinelli Rd
North Bethesda, MD 20852

WEBCASTING INSTRUCTIONS: For the optimal webcasting experience, please ensure you have the Silverlight Plugin. You can obtain this free plug in [here](#). Also note that users will be able to watch the archived webcast on PCs and Macs, as well as on mobile devices such as iPads, iPhones, and Droid Phones.

Welcome

(Karen Midthun, M.D., Acting Director, Center for Biologics Evaluation and Research, FDA)

Meeting Overview

(Celia Witten, Ph.D., M.D., Director, OCTGT, FDA)

Session 1

Cell and Gene Therapy Clinical Trials in Pediatrics: Regulatory Oversight and Research Ethics (Moderator: C. Witten)

- **FDA IND Review: Regulations and Challenges**
(Wilson Bryan M.D., Clinical Evaluation Branch Chief, OCTGT, FDA)
- **Ethics of Pediatric Clinical Research**
(Steven Joffe M.D, MPH, Assistant Professor of Pediatrics, Harvard Medical School)
- **The Ethical Path Forward for Pediatric Product Development**
(Robert Skip Nelson M.D., Ph.D., Pediatric Ethicist, Office of Pediatric Therapeutics, OC)
- **Question and Answers**

Session 2

Clinical Trials of Cell and Gene Therapy in Pediatrics: Examples from the Clinical Research Community (Moderator: George Daley, M.D., PH.D. Samuel E. Lux, IV Professor of Hematology and Director, Stem Cell Transplantation Program, HHMI/Children's Hospital Boston, and Professor of Biological Chemistry/Molecular Pharmacology and of Pediatrics, Harvard Medical School)

- **Clinical Trials of Cell Therapy in Pediatrics Example**
(Helen Heslop, M.D., M.B., Ch.B., Professor, Department of Pediatrics and Medicine, Baylor College of Medicine)
- **The Development and Translation of Tissue Engineered Vascular Grafts for use in Congenital Heart Surgery**
(Christopher Breuer, M.D., Associate Professor of Surgery (Pediatrics) and of Pediatrics and Director of Tissue Engineering, Yale School of Medicine)

BREAK

- **Gene Therapy for Pediatric Immune Deficiencies**
(Donald Kohn, M.D., Professor, Microbiology, Immunology & Molecular Genetics and Pediatrics, University of California at Los Angeles)
- **CNS Gene Therapy in the Pediatric Population**
(Ronald Crystal, M.D., Professor of Genetic Medicine and Chairman, Department of Genetic Medicine, Weill Cornell Medical College)

Panel Discussion on Session 2 (Chair: George Daley, MD. PhD)

Members: Helen Heslop, MD, MB, Ch.B, Christopher Breuer, MD, Donald Kohn, MD, Ronald Crystal, MD, Theresa Chen, PhD., Pharmacology/Toxicology Reviewer, DCEPT Pharmacology/Toxicology Branch, OCTGT, FDA, David Maybee, MD, Medical Officer, DCEPT Clinical Evaluation Branch, OCTGT, FDA

LUNCH

Session 3

IRB oversight (Moderator: Jeffrey Botkin, MD, MPH, Professor of Pediatrics, Department of Pediatrics, Adjunct Professor of Medicine, Department of Internal Medicine, University of Utah School of Medicine and Associate Vice President for Research Integrity, University of Utah)

- Advocating for pediatric subjects on the IRB
(Angelica Walden, MBA, Patient Advocate, Department of Quality Management, Medical College of Georgia and Institutional Review Board Member, Medical College of Georgia)
- Ethical considerations in pediatric gene therapy trials--Perspectives from the RAC
(Jeffrey Kahn, PhD, MPH, Director, Center for Bioethics, University of Minnesota and Professor, Department of Medicine, University of Minnesota School of Medicine)
- IRBs and Subpart D: A Practical View From the Field
(Susan Kornetsky, MPH, Director of Clinical Research Compliance, Children's Hospital of Boston)

BREAK

Session 3 Continued

- Subpart D: View from one IRB
(Scott Denne, M.D., FAAP, Professor, Department of Pediatrics, Indiana University School of Medicine)

Panel Discussion on Session 3 (Chair: Jeffrey Botkin, MD, MPH)

Members: Angelica Walden, MBA, Jeffrey Kahn, PhD, MPH, Susan Kornetsky, MPH, Scott Denne, MD, FAAP, Robert Skip Nelson MD, PhD, Jerry Menikoff, MD, JD, Steven Joffe, MD, MPH

Wrap-Up and Closing Remarks (Celia Witten, PhD. MD)